

**METHODS AND COMPOUNDS FOR MODIFICATION
OF ABERRANT OR ALTERNATIVE SPLICING OF PRE-MRNA**

ABSTRACT

The present invention provides a method of preventing a splicing event in a pre-mRNA molecule, comprising contacting the pre-mRNA and/or elements of the splicing machinery with a small molecule compound identified according to the methods described herein to prevent the splicing event in the pre-mRNA molecule. Further provided is a method of inducing a splicing event in a pre-mRNA molecule, comprising contacting the pre-mRNA and/or elements of the splicing machinery with a small molecule compound identified according to the methods described herein to induce the splicing event in the pre-mRNA molecule. Furthermore, a method is provided herein of treating a patient having a disorder associated with an alternative or aberrant splicing event in a pre-mRNA molecule, comprising administering to the patient a therapeutically effective amount of a compound identified according to the methods described herein to prevent an alternative or aberrant splicing event in a pre-mRNA molecule, thereby treating the patient.